Vertex Pharmaceuticals:

An Application of Modern Pharmaceutical Strategy & Valuation Methods

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I. Executive Summary

This project applies strategy frameworks and valuation methods to Vertex Pharmaceuticals to present tactical recommendations as would a consultant in the pharmaceutical and biotech industries. In addition, it creates a trading recommendation based on a financial valuation projection model. It achieves both of these by first describing the company’s context and then using frameworks as a lens to analyze the firm, industry, products, customer needs and regulatory environment. The next section includes a discussion of various pharmaceutical valuation methods and applies one to Vertex. Some expository follows covering a recommended trading decision based on the analysis and considerations for the longer-term future.

II. Purpose

Vertex pharmaceuticals is the leader in medication treatments for cystic fibrosis, a severe life shortening disease caused by a family of genetic mutations that affect the lungs and breathing. The company already serves about 45% of the global market with their two marketed drugs, KALYDECO® and ORKAMBI®. They have recently gained FDA approval for a double combination therapy which will begin selling in 2018. The pipeline product of most interest is a triple combination therapy currently in late stage clinical trials, that if approved, would allow them to serve 90% of the global CF patient market. This makes Vertex a very interesting company to analyze.

Analyst opinions differ greatly over Vertex’s value. It currently trades at more than 50 times earnings and is now profitable for the first time in 2017. The blockbuster revenue potential the firm maintains will only be realized for shareholders if they can launch efficacious pipeline products, secure payer coverage worldwide and partner with patients to assist them along their disease journey.
III. Research Questions

This paper investigates and answers the following research questions:

1. What is the current strategic direction of Vertex Pharmaceuticals? How does this fit into the context of the 2018 pharmaceutical/biotech industries? Which tactical strategies can be implemented to improve the firm’s execution of goals and performance?

2. What are the biopharmaceutical valuation methods? Which one is best and for whom?

3. What is the value of Vertex? Is the recommended trading decision buy, sell or hold?

IV. Methodology

The methods used to analyze Vertex include careful study of industry reports, current events, company filings and announcements in order to determine strategic objectives. The frameworks used are *The 4Ps of Marketing* to analyze the company’s micro environment and Michael Porter’s Five Forces to analyze the macro environment trends. Framing the company with these paradigms gives insight into the major challenges of the firm and elucidates targets for potential action to fix them. The recommendations that stem from these analyses present viable solutions to the problems.

The valuation portion of the report analyzes the literature to build a financial model that contains assumptions based on the industry and company research, history, and disclosed company investment projects. The projection includes both a scenario (low, average, high) analysis as well as a sensitivity analysis that shows which variables are likely to create the largest projection changes. To do this, the model adds projections for the current company with a terminal value, the recently approved SYMDEKO® and a triple-combination therapy in development.
The current company value is forecasted using a Free-cash-flow (FCF) method which requires a 5-year pro-forma projection built off the previous five years of quarterly financial data. A terminal value is also estimated using an assumed perpetual growth rate. All of the cash flows are then discounted and summed to arrive at the present value of the company. The discount factor used is the weighted average cost of capital discount rate (WACC) because all of the expected firm cash flows have the same business risk and leverage as the firm itself. For any flows that do not fit these criteria, another project or divisional discount rate should be used. WACC is estimated using the CAPM model and average corporate bond interest rates.

The product, SYMDEKO®, is valued according to a simple discounted cash flow (DCF) method. Here, assumptions about the market size and price are given by research and historical precedent to predict the cash flows which are then discounted by the appropriate rate.

The triple-combination therapy is valued according to a Monte Carlo Simulation which accounts for a probabilistic range around the input variables and returns a distribution of outcomes. This value is incorporated into the enterprise value using different scenarios: mean, low, and high at a 90% confidence interval.

An implied share price is then calculated based on the valuation given by the above methods and a trading recommendation is given after comparing this result with the current market share price.

The model is interactive so that the reader may modify or update any assumptions to view the effects of the change on the projected value and returns. The DCF method is compared to other ‘back of the envelope’ valuation methods including using the industry multiple method.
V. Data

The report uses data and research from a multitude of sources. A summary of the data sources is given in the table below.

<table>
<thead>
<tr>
<th>Data</th>
<th>Source/Type</th>
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<tr>
<td>Financial Data</td>
<td>Company 10-Q Filings, Bloomberg5</td>
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<tr>
<td>Market Data</td>
<td>Analyst Reports, CF Foundation, Journals</td>
</tr>
<tr>
<td>Product Data</td>
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<td>Industry Reports</td>
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<td>Pricing &amp; Timeline</td>
<td>Firms Executives, CF Foundation, History</td>
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<td>Empirical, Survey Data</td>
<td>PubMed, Journals</td>
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VI. The Company

a. Industry Overview

The Pharmaceutical and Biotechnology industries are players in the healthcare value chain whose mission is to discover, develop and commercialize medicines. This paper will use the terms pharmaceutical and biotechnology or biotech fairly interchangeably. A classical distinction is made between pharmaceuticals and biotech where pharmaceutical companies are focused on chemical-based treatments whereas biotech is focused on biologic molecule development. However, most modern companies in the industry are found somewhere in-between these two definitions and are best considered as ‘biopharmaceuticals’. As such, either term will suffice to refer to companies that develop and market prescription medications.
Nearly one trillion dollars are spent on medications each year which accounts for a significant portion of global healthcare spending.\(^1\) The industry has grown since the first organizations launched and the demand for medicines continues to expand as populations rise and understanding of treatment advances. The companies in these industries are characterized by long investment horizons due to the lengthy and extremely risky drug development process. However, once approved, launches have massive potential for success because of the patent protection issued for new drugs. This incentive propels firms to undertake the massive investment risks required to develop novel medicines and therapies.

There is a large current trend of early-stage firms which are often born out of academic laboratories as a result of the efforts of the many distinctive scientists researching there. These biopharmaceutical firms focus on treatment of niche, ‘rare’ diseases that have less than 100,000 patients and are now treatable because of noteworthy advances in precision medicine. They are often able to garner the backing of venture capital investors who incent these companies to eventually IPO or exit by becoming acquired. As a result, there is a ton of merger and acquisition (M&A) activity in the field as larger companies seek to add to their drug pipeline portfolios by buying the research expertise and intellectual property held by others.

\[\text{b. Company Background}\]

Vertex Pharmaceuticals is a fully integrated research and commercial medicine developer focused on developing therapies for “serious” diseases. Vertex was founded in 1989 by Joshua Boger and found a niche in the gap between biotechnology and traditional large pharmaceutical firms. As previously mentioned, the biotech industry was classically characterized by intense research in drug discovery that

\(^1\) (Berkrot, 2016)
involved biological compounds such as bacteria or enzymes in contrast with the pharmaceutical companies’
chemical-based drugs. Boger was a leading researcher at Merck before founding Vertex and had significant
experience designing research programs and successfully developing and commercializing products.

At Vertex, he instilled a culture of research and innovative science. This culture often created
“analysis paralysis” where no hasty decisions were made until all of the evidence was known and complete.
The company utilized a unique method to screen for drug candidates by starting with knowledge of the
cause of the underlying disease and then searching for compounds that have possible indications on the
defective proteins or enzymes. In contrast, most drug companies used High-Throughput Screening to
rapidly assess thousands of candidates to determine which ones may be viable, then looked for a disease to
which they could apply them. Vertex developed and licensed several products to larger portfolio
pharmaceutical firms which eventually saw their patents expire in the subsequent years. In the 2010s, there
was a shift from this model of research and licensing to developing and selling their own product line. The
company revenues are now almost entirely dependent on the Cystic Fibrosis (CF) treatment products.

Vertex is based in Boston, Massachusetts which is a major biotechnology innovation hub due to
the many academic research institutions in the area. They serve as a major source of scientific talent for the
companies. The firm also has labs in California which is renowned for its technology focused culture.

c. Cystic Fibrosis

Cystic Fibrosis is a disease caused by a mutation in the CFTR protein gene. This mutation causes
the patient to have defective or non-existent CFTR proteins in their lung cells. The result is the inability of
the lung cells to transport salt and water across the membrane leading a build-up of mucus in the lungs that
makes it hard to breathe and life much more difficult. Ultimately, death occurs due to lung damage. Though
life spans for CF patients have improved thanks to improved treatment protocols and new medicines, the
median life span is still only 29 years.
There are over 2000 known mutations of the CFTR gene that cause CF. Each case requires both standardized and customized treatments to help patients live longer better lives. To obtain this disease, children must have inherited two defective CFTR genes from each of their parents. The disease affects primarily Caucasians with an incidence rate of approximately 1/2500. There are approximately 75,000 cystic fibrosis patients in North America, Europe and Australia. 30,000 are estimated to be living in the United States based on figures from the Cystic Fibrosis Foundation’s disease registry.

As recently as 50 years ago, little was known about the root cause of CF and patient outcomes were extremely poor with most not surviving to adulthood. Treatment typically involved daily “thumping” on the patient’s back to loosen and help cough out the excess accumulated mucus. CF treatment programs began becoming more commonplace at hospitals across the U.S. and new inventions like steam tents for patients to sleep in were created to help prevent the mucus build-up.

The discovery of the CFTR protein, the underlying cause of CF, gives much hope that there will someday be a cure. This leads back to Vertex who is the leading manufacturer of treatments that target the deficient CFTR protein. These drugs in combination with other symptom reduction treatments has significantly helped patients breathe better and live better lives.

VII. Products

Vertex has a long history of developing treatments for a wide range of conditions including HCV, Rheumatoid Arthritis, and heart conditions. However, the current strategy is primarily focused on treatments for Cystic Fibrosis. The company currently has two commercially available products, with a third that was just approved in 2018 with launch early this year. The existing treatments were approved for about 45% of all CF patients with this likely to rise further due to the new approval.

2 (Scotet, 2012)
3 (Cystic Fibrosis Foundation, 2018)
a. **KALYDECO®**

The first product, ivacaftor (KALYDECO®) is part of a class of drugs called potentiators that help keep the defective CFTR proteins open and near the surface of the lung cells so that it can help clear mucus through the transport of salt. It is taken twice orally each day by patients and was the first approved medicine to treat the underlying cause of cystic fibrosis. It came to market five years ago in 2012 and the company’s strategy for the product has been to continue to expand the label indications so that more patients with different mutations can benefit from this drug. This is done by continued research studies and new data. In 2017, the FDA approved KALYDECO® for use in five additional mutation indications allowing 600 more patients to gain access to the drug and 2017 product revenues to reach approximately $800 million.\(^4\)

b. **ORKAMBI®**

The second drug, ORKAMBI®, is a combination therapy pairing lumacaftor and ivacaftor.\(^5\) The former fixes the defective CFTR protein in a way that allows it to be pushed to the proper place on the cell surface. The latter functions as mentioned previously to open the CFTR protein and keep it stable at the surface to facilitate the transport of chloride. This combination drug only treats patients 6 and older that have the F508del mutation which is the most common CF mutation.\(^6\) This population is approximately 11000 people in the United States. It is also taken orally each day. The 2017 product revenues were about $1.2 billion. There is nearly full uptake for the F508del population older than 12 (8500 patients) and close to maximum uptake for the 6-12 year old population that was approved in mid-2016. The continuing strategy relies upon approval of reimbursement agreements in Europe, in which Vertex made deals in Summer of 2017 with Italy and Ireland. Only around 60% of the patients in European countries have full access to the treatments. One particular struggle for Vertex has been gaining payer coverage in France.

\(^4\) (Vertex, 2016)
\(^5\) (Cystic Fibrosis Foundation, 2016)
\(^6\) (Cystic Fibrosis Foundation, 2017)
where they have been selling products, but have not yet recorded any revenues due to not being able to reach an agreed upon reimbursement price with the government.

c. **SYMDEKO®**

Going forward, Vertex seeks to continue its development in the CF space. There is currently a double combination therapy of Tezacaftor (VX-661) and ivacaftor (KALYDECO®) that has undergone FDA review and been approved. It is being launched under the brand name SYMDEKO®. They had priority review and extremely positive phase 3 data that led to expectation of a positive a decision that was announced in February 2018. This therapy is hailed as a ‘next-generation’ protein modulator and has shown significant improvement in mucus clearing. It is also likely to cannibalize some sales of KALYDECO® because of the additional benefits and some overlap in the patient populations that it is approved for. However, the expected expansion of treatment will far outweigh this in the long-run.

**VIII. Context**

a. **Pipeline**

The most exciting part of the pipeline for investors are the several triple-combination CF therapies in development. These have all shown very positive top line data and have indications for patients with only a single F508del mutation. If one of these therapies is approved in the coming years, Vertex will have drugs that can benefit over 90% of all cystic fibrosis patients. This is a massive increase in their market share. It has implications of doubling or more their revenues. Vertex hopes to submit an NDA for one of these by 2021, but will require a few more years of intensive research and spending to advance the pipeline. Vertex has an important partner and investor in the Cystic Fibrosis foundation. The initial funding for the CF program development was funded by this group that was founded by parents of patients to research new therapies. Biotechnology companies need large amounts of financing and this advocacy organization partnership represents a new model of gaining the necessary funds.
b. **Drug Development & Regulatory Approval Process**

Critical to the understanding of the industry are the challenges of the drug development and approval process. Once a drug candidate has been identified, it must undergo extensive testing and the approval process (FDA in the U.S. and EMA in Europe) before being offered commercially as a product. Most drugs never make it into development because they not only need to have evidence that it works, but also reasonable commercial viability in order for the company to invest in the costs of development. It is estimated that a new drug costs upwards of $1 billion in development spending and takes 7 – 15 years to be approved for marketing. In the U.S., new drugs must pass the clinical trial process by testing and presenting data to the FDA. They must meet the requirements for efficacy and safety through implementation of the following research and development process:

- **Phase 0/Pre-clinical** – Small dosage tests in humans, unlikely to benefit them
- **Phase 1** – Is it safe? What dosage is best?
- **Phase 2** – Is it effective for the diseases being studied?
- **Phase 3** – Is it better than other products? Either more effective or less side effects in a randomized controlled trial (RCT)
- **FDA Approval** – Is the data showing a favorable risk-benefit profile that would give reason for approval

c. **Regulatory Environment**

After the election of Donald Trump as President in 2016, he rolled out a ‘100-day action plan’ that included a proposition to streamline and speed up the drug approval process at the FDA. Pres. Trump then appointed Dr. Scott Gottlieb as head of the FDA. This bodes well for the pharmaceutical industry because Dr. Gottlieb has extensive background working in pharmaceuticals and health policy. He has hinted at a
new initiative to allow ‘real-world data’ in the consideration of approvals. This is a marked change from previous processes that rely heavily on only clinical data. Though Gottlieb will seem to allow for flexibility in the process, he comes from a rigorous scientific background that will hopefully serve well to protect the legitimacy of pharmaceutical products by protecting patient interests⁷.

IX. Analysis I

The following analysis applies several business frameworks to Vertex to more clearly define and understand the current competitive situation. This is done because the frameworks serve as a lens to more accurately diagnose the current scenario of Vertex. From this analysis, several tactical recommendations will emerge as changes or additions to the strategic plan. The paradigms used for this analysis are Porter’s Five Forces to investigate the effect of macro factors that the company can affect through strategy, and the 4Ps of Marketing that show the micro factors over which Vertex has direct influence.

a. Macro Environment

This section uses the Porter’s Five Forces framework to investigate the macro environmental factors surrounding Vertex.

Rivalry

Some context has been discussed previously with regards to how the industry functions and Vertex’s place within the biopharmaceutical industry. They currently have the only approved treatments for treating the root-cause protein deficiency that causes CF and enjoy a monopoly. This however, will not last because the lucrative CF market will attract many new companies looking to grab some market share. The incidence of the disease is not expected to grow because

⁷ (Clarke, 2017)
it is genetic and does not spread or get passed on with greater frequency. Market share is even more important with this in mind as the approximately 75,000 patient population will not grow larger overall. This is the case with many ‘rare’ diseases where there is a race between firms to develop a better product and serve previously unmet medical need. There is little in terms of price wars as it is disguised throughout the healthcare system. With the internet and services like WebMD, more patients are taking care into their own hands and although doctors are the only ones that can prescribe a medication, brand perception among end consumers is increasingly important. The successful companies going forward will those who are able to adopt new technologies (Artificial Intelligence, Virtual Reality, Machine Learning) and have a clear understanding of patient needs.

**Threat of Substitutes**

There is little distinction between rivalry and substitutes in this industry while a drug is still on patent. Once the patent expires and generic versions of the drug are produced a price-war begins as the differentiating factor of disease improvement is now easily replicated. Interestingly, the field of bio-similars is emerging which are in essence the generic versions of biologic protein therapies. There is a provision under the FDA guidelines to accelerate the approval for certain bio-similar products and allow these manufacturers to circumvent patents with only small changes in the molecule structures in hopes of providing more affordable therapies. However, adoption of these treatments (only 7 approved at time of writing) has been extremely slow both because payers do not trust the non-brand name to provide the same level of efficacy and safety as the precedent medication and because doctors hesitate to switch a patient off of a drug that is working well for a chance at saving money. Firms that produce treatments for these rare diseases should be cautious of bio-similars in the event that the healthcare system starts accepting them more.
Vertex has strength in research and development due to their history as a research focused organization. Their unique drug candidate identification approach allows them to more quickly discover and produce effective biologic molecules. This will help in the long-run against potential substitutes because their manufacturing capacity can be flexed to produce more cheaply or even to extend patent life by slightly modifying molecules. One weakness is their current strategy because revenues are mostly dependent on Cystic Fibrosis as the only disease area. This exposes them to significant risk if a competitor were to enter and break the monopoly thereby grabbing a large portion of market share. The firm also has strong a strong pipeline which includes more treatments for CF, Pain, Cancer, Influenza and a partnership with CRISPR Therapeutics to use their technology to develop gene-editing treatment for sickle-cell disease. Vertex also has a strong commercial team as evidenced by the high level of uptake for patients in markets that have coverage. The strong brand recognition and overall favorable PR view on Vertex gives the company tremendous opportunity.

**Threat of New Entry**

Vertex has long held a monopoly on root-cause CF treatments. They will continue to hold this, but there are other companies hot on the tail who are lured by the high reimbursement prices even though the target market is a fairly small rare disease. Galapagos NV has announced that it has positive data for one piece of its triple-combination therapy that is in development. They are seeking a partnership with AbbVie to help with funding this project. Protalix also has a drug in phase II development called PRX-110, that is aimed to have mucus clearing benefits for all CF patients because it does not treat root cause. It is seen as a potential competitor to KALYDECO®, but the more recent data has shown it to be less effective than originally thought. Both of these competitors present a major threat the monopoly that Vertex currently holds. At any point, a competitor that brings to market a product that treats mutations that Vertex does not already cover or that shows greater efficacy with fewer side-efffects will massively affect the projected sales. In general, most firms will not be able to acquire the capital and intellectual property needed to enter the market, but some will with enough time and resources.
Suppliers/Collaborators

Vertex is itself the manufacturer and developer of its products. It does not have much reliance on outside suppliers because it can make its own drug products and does so at a reasonable cost. The firm does have several key collaborators to consider. The first, are the payer systems and governments that regulate the prescription medication industry. The firm uses its relationships with these groups to negotiate reimbursement prices for the products, thus giving financial incentive to invest in development and provision of the therapy. Market access in Europe has been a long battle for Vertex in getting their CF products covered under insurance. There have been particular challenges to gaining coverage in France. The second, is the Cystic Fibrosis Foundation (CFF) which as an advocacy group is able to utilize their influence to assist patients and to facilitate access to beneficial treatments (including Vertex products). This partner is an important resource for Vertex because it provides patients with information and helps Vertex with unique methods of financing due to their status as a non-profit third party entity. The CFF supports all treatment efforts, including those of Vertex competitors, and as such Vertex should seek to constantly foster a positive relationship with the group to continue to help increase the access to treatment regimens that include Vertex’s products.

Other collaborators include large pharmaceutical companies that have licensing deals with Vertex for promising early-stage treatments who have the commercial rights to sell the product if it reaches approval. Vertex also has several deals of their own with investigational research groups in which they will pay milestone payments for phases attained in development and be responsible for commercialization should any of the molecules be approved.

Buyer Power

The end customers for Vertex are the CF patients and their care-givers/families. The disease is genetic and as such is unlikely to grow as a total market but continuing to expand the label for the available
drugs will help patients obtain life-altering medication. CF patients are excited about the opportunity to further improve their condition with the upcoming next-generation treatments and the newly approved SYMDEKO®. Vertex has filed for regulatory approval for SYMDEKO® with the EMA for marketing in Europe so that thousands of patients can get treatment for the first time. Access is a major factor for Vertex customers.

The customers paying the real price of the products are the payer groups. A major concern for access is the changing state of the healthcare value chain in terms of how payers (insurers) now prefer to reimburse based on outcomes. Also, there are changing patient preferences for how they receive treatment in that they prefer to receive a full set of comprehensive services and support like they do for other products. With new technology that allows for tracking a patient all the way through their journey from diagnosis, to treatment and the resulting improvements, insurers seek to leverage that advanced data to only pay in accordance with the ultimate outcome level. This makes it critical for Vertex that the drugs have strong effect on improving breathing capacity and that adherence to the exact regimen once prescribed is followed.

One major issue that arises in daily oral medication is that of adherence to the treatment regimen. Additionally, since many CF patients are children they rely upon their parents or care-givers to get them to and from doctor visits, administer thumping on their back, and keep track of the medication use. This becomes a difficult task and people often forget when they last took their medication. To help solve this, Vertex has an extremely strong patient access and support services division. One tactic they are using to promote greater adherence is by partnering with PCI Services to deliver the months treatment dosages in a special box. The packaging clearly displays key information about dosage timing, has dividers for each week and custom labels for morning and night doses so that patients can more easily remember when they last took the medication. It is shown in the figure below.

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The patient access team also helps by assisting with financially distressed families who cannot afford treatment, expanding the number of locations and ways where the drugs can be picked up, and by helping patients get to experienced providers who are experts in CF. They facilitate conversation between the doctors and patients to help monitor the treatment protocol and guide them to experts for any questions or concerns regarding the medication and its effects. Patients’ health relies upon Vertex and it is critical to their mission and to the goal of treating CF to support them at all stages of their journey through care. Customers more than before demand ultimate service and transparency of their treatment.

Source: Pharmaceutical Commerce
Conclusions of Porter’s Five Forces

The five forces analysis can best be summarized as displaying that Vertex currently holds a strong position in the CF disease area, but they are subject to a number of risks that may disrupt that position. These include reimbursement challenges and new entries to the market.

b. Micro Environment

This section performs an analysis of the micro-environment of Vertex. Specifically, it uses the 4Ps of Marketing framework to investigate the internal company-controlled factors.

<table>
<thead>
<tr>
<th>Price</th>
<th>Product</th>
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<td>• $270 – 311k</td>
<td>• CF Drugs, oral</td>
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<tr>
<td>• Locked in once</td>
<td>• Rare disease</td>
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<td>• Minimal, serious</td>
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<tr>
<td>• Mail delivery or</td>
<td>disease sells</td>
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<tr>
<td>pickup</td>
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Source: Own Creation

Product

Vertex exclusively sells medication for Cystic Fibrosis. The current medicines are taken orally to improve breathing by treating the underlying cause of the disease. The company also looks to expand their offerings to other rare diseases.
Price

The Vertex products achieve fairly high reimbursement prices with ORKAMBI® priced at $272,000 per patient per year, KALYDECO® at $311,000 per patient per year and the newly approved SYMDEKO® $292,000 per patient per year. Though these prices may seem high as a sticker price, they are actually within reason for the current healthcare system because the insurers are the ones paying most of the cost. These payers agree to the prices upon the drug launch after negotiating with the producer about the total social benefit that each treatment will create. This benefit measure includes things like quality of life measures and increased productivity for both patients and their families. Once the price is negotiated, it is locked in and neither party can change it until the length of the agreement ends.

Place

Vertex products are prescription only medications and as such are subject to the rules and regulations of each country that they are selling into. The products are delivered to patient’s homes directly via mail or can be picked up at a specialty pharmacy.

Promotion

The firm does little in terms of promotion. This is partly because there are not yet other competitors on the market, but more so the nature of the target condition. A serious chronic disease like CF demands treatment regardless of additional promotions.

Conclusions From 4Ps

Product is the most important of these micro factors because people only want the medication if it is safe and if it works. Medicines with potentially severe side-effects are not tolerated well in the market, even for people with serious diseases. Also, the drug must be helping to improve symptoms, or it will not
be prescribed. So far, Vertex has done well by creating products that help some CF patients breathe better and have minimal side effects. Price is the second most important because it serves as an important factor for access within the healthcare ecosystem. If payers do not adequately cover the costs, patients will not go on drug, and no sales will be made for Vertex. Place and promotion are less important as the prescription drug system cannot be altered and sick patients demand the medication regardless of additional promotions.

c. Recommendations

From these analyses emerge several recommendations for Vertex to improve their position. As demonstrated by the Porter’s Five Forces analysis, the threat of new entry is extremely potent. To help mitigate the loss of market share upon a new entrant’s arrival the firm should seek to guard against competition by developing brand perception as a patient inspired firm. One way that they might achieve this is by providing industry leading patient support services. This could be in the form of new adherence protocols because even with a such a serious disease as CF, there is a high rate of non-adherence to the daily oral drug regimen. The company can provide a daily text message or a mobile application to remind patients to take the medication. They can also seek to partner with a diagnostic company to determine the level of drug in a patient’s system at a given time so that they know when to take the next dose. Vertex may support patients by establishing a college support fund because many of the patients on the medication are young and with the help of the treatment are living longer lives and wish to pursue higher education. Lastly, continuing to the build out the online patient services cloud adds value by allowing patients and their medical providers to communicate directly with each other to track the treatment protocol. This notion of pharmaceutical companies providing patient support services is a developing trend in the industry, but one which is necessary for success. The additional value they create is important along with creating efficacious products and filing strong intellectual property patents.
Vertex should also seek to continue to create access to their products for eligible patients. This may entail generating deep health economic (HEOR) data to gain payer coverage in the EU. This HEOR data shows the efficacy of the products and analyzes the social benefit versus the cost of the drug. The firm should also continue to negotiate strong, but justifiable reimbursement prices as they have done for their products so far. Anecdotal evidence can also be a powerful tool in gaining coverage. One tactic they may also look to employ is to provide discounted genetic testing to confirm which mutations that the patients have and subsequently start them on the appropriate drug if eligible. A further application of this technique using new technology would be to employ Artificial Intelligence (AI) on registry data from the Cystic Fibrosis Foundation. This would analyze family trees and preemptively predict which people will have the disease as new form of precision medicine.

Source: Deloitte University Press

Additionally, to create more access, Vertex should seek to develop novel systems or partnerships to help the under and uninsured get the life-extending medication. They have a lot of flexibility in how this is implemented because the CLV of a customer is estimated to be around $10 million and spending anything up to that amount to get someone on Vertex products is thus a worthwhile investment.

Vertex should seek to de-risk the company by expanding outside of CF as the only disease area. To do this, they should seek to acquire to build R&D capacity for products that fit their existing strengths around selling products for debilitating chronic conditions with a minimal sales force. Deep analytics is the future of the industry and should be used to identify patient needs and add-value by catering to them. Lastly, Vertex can create positive PR by partnering with advocacy organizations like the CF Foundation. They also serve as a unique source of financing because those non-profit entities have access to funds that they can then lend to the drug manufacturers on favorable terms since their interests are not purely financial and can indefinitely delay the payback period.

X. Analysis II

This section first shows the past investment performance of the VRTX stock and continues with a discussion of various biopharmaceutical valuation methods and their associated literature. It then posits a simplified version of the LSM Monte Carlo method as the preferred choice for pharmaceutical pipeline valuation and applies the method to Vertex. Further analysis continues by viewing Vertex’s potential as an investment and recommends a trading decision.

The valuations in this analysis are rooted in corporate finance principles. It presents a method of projecting quarterly pro-forma cash flows for the next 20 quarters using a set of assumptions based on historical and future trends. It invokes the Free-Cash Flow (FCF) method where the value of a firm is determined by analyzing the proportion of discounted cash flows going to equity-holders versus bond-holders. It sums the projected discounted cash flows from the
currently available products as well as the expected NPVs of the recently approved SYMDEKO® and the next generation triple combination treatment currently in Phase 3 studies. This product combines the already approved tezacaftor and ivacaftor along with a new compound, VX-659. All the cash flow to equity is considered as part of the returns of the firm and a target stock price is determined by dividing the value of a firm’s equity by the number of shares outstanding. The multiple methods discussed in the next section are frequently used by the industry to project the value of the pipeline products amidst a set of uncertain conditions including contextual, technical, and commercial challenges.

a. Past Investment Performance

Vertex’s stock currently trades at $161.89 as of day close April 12, 2018, which is more than 50 times its earnings. This is common among biotechnology stocks that have some indices citing industry benchmark P/E ratios of more than 100.\(^\text{10}\) The following figure displays the stock performance over the previous five years.

\(^{10}\) http://pages.stern.nyu.edu/~adamodar/New_Home_Page/datafile/pedata.html
This shows a return of approximately 124% over five years and approximately 52% over the past three years. This is compared to a biotechnology performance index called ARCA of which Vertex makes up 3% of the composite.

This shows an approximately 126% five-year return and approximately 13% return over the previous three. This puts the Vertex stock on par with the industry as a whole and shows that it is now greatly outperforming (39% better). This is attributable to having three approved products on the market, two of which were approved in those previous three years from 2015 to 2018.

b. **Discounted Cash Flow (DCF) Model**

The first valuation method discussed is the discounted cash flow model (DCF) net present value approach. Here the cash flows of a project are projected for the entire time that it runs and discounted back at some discount rate \((r)\), that accounts for the time value of money and risk of the project. This method is fairly straight-forward and easy for managers to implement. They must simply estimate the investment required, market size, segment size and price to determine future
cash flows. All uncertainty or risk related to the project is assumed to be picked up by the discount rate. However, this is unwieldy in pharmaceuticals/biotech because of the extremely long investment horizons (7-15 years to develop a drug) and the high risk of technical or commercial failure.\textsuperscript{11}\textsuperscript{12} ENPV methods only consider risk and managerial flexibility as assumptions based on the probability of technical success or failure in the drug development process. In real portfolio management decisions, there is a managerial option to abandon a project which is discussed in the next section.

c. Real Option Valuation (ROV)

Real options are investment projects that have the option to abandon or continue a project based on the progress and economic conditions around it. This flexibility negates much risk of a bad investment because firms can cut their losses once there is evidence that it may not be worth it to pursue further development of the project. This is much closer to a drug development project where, for example, poor safety data on patients testing the drug in Phase II development stage could cause the management to abandon the project and divert resources towards other more promising candidates.

\textsuperscript{11} (Park & Shin, 2018)
\textsuperscript{12} (Willigers, & Hansen, 2008)

The ROV method has been a standard in pharmaceutical valuation as it is still relatively accessible to analyze from a manager’s point of view and still incorporates many of the important conditions surrounding the success or lack thereof of a new drug launch. The technique developed by scholars is a binomial lattice as presented in the above figure. The model accounts for upwards and downwards trends at each decision node with a certain probability assigned for each state. If a project’s net value is negative along a given path, it is typically abandoned. The estimated value of the project will be higher compared to the DCF model because of the flexibility to adjust based on incoming data is a benefit to the cash flows. The sources of uncertainty accounted for are both technical and market risks. This ROV method has been used in many valuations over the last few decades and further modifications to it have been developed such as risk-adjusted Net Present Value (rNPV) which uses probability factors to determine the expected value of the project. However, this model has limitations in that it is unable to consider unforeseen or unmeasurable uncertainties.
d. Monte Carlo Simulation

To address the concern that all options, known and unknown, along a proposed project path are addressed, several studies propose a Monte Carlo Simulation (MCS). This is a stochastic process model that is able to accept a range and distribution of input variables, run thousands of ‘scenarios’ driven by some volatility factor(s) to return a mean and distribution of expected outcomes. Willigers et. al and Park & Shin offer the technical understanding about the various methods that overlay the theory of ROV with the more robust MCS models and how they differ from the standard model using common index data and a composite of pharmaceutical data. Park & Shin suggest that the regime changing (MRBL-MRS) model is the best because it accurately represents the effect of mean reversion over the lifecycle of a product. Additionally, it claims to consider the effects of regime change (of government) with different preferences for the regulation of the drug development industry.

This technique works well, however, it is only practical with the perspective of an R&D Portfolio manager. For these folks, their primary concern is to deliver economic returns on the R&D investment spread and optimize it for the most promising candidates. In contrast, investors should look to use the simpler GBM or Mean-Reversion (MRP) instance of the Monte Carlo technique. This considers only one or two volatility factors respectively and is much more practical for the investor to build, but still robust enough to show accurate projections. This type of analysis allows for inputting a fairly wide range around the base assumption where then the model can pick up that variance in outcomes.
XI. Valuation

The following presents an application of the aforementioned valuation techniques to estimate a share price for Vertex. It does so in three parts, the first being a pro-forma projection of the current company, the second a DCF valuation of SYMDEKO® and lastly an applied Monte Carlo Simulation to the furthest developed pipeline product. These are all considered together as the value of Vertex.

a. Capital Structure & Financing

The Vertex capital structure is financed by 53% equity and 43% debt. In the biopharmaceutical industry, financing the large and continuous R&D expenditures needed for new molecule discovery is critical to success. There is a constant search for new investors and unique licensing agreements with a range of various stakeholders including academic research institutions, CROs, big pharma, and other peer biotechnology firms. The types of licensing deals that arise are often complex and unique to each product as is the case with Vertex. Some deals only license the commercial rights to a drug in certain countries or regions where a partner company may already have a well-developed commercial sales team in that particular therapeutic area. Still others are built on milestone payments where each round of investment is predicated on advancement through the development pipeline.

b. Discount Rate

The discount rate is used to calculate the present value of future cash flows. It is also a measure of risk of a project. The discount rate used in the valuation of Vertex is calculated by the Weighted Average Cost of Capital (WACC) which is the discount rate of the firm. It is derived by the CAPM method which accounts for the exposure to the systematic and unsystematic risk of the firm. The firmwide WACC is used to evaluate the future cash flow projections from Vertex’s cystic fibrosis projects because those projects are the same business of the firm itself and have an almost exact riskiness as the operations of the firm.
calculations for this value can be found ‘FCF Valuation’ tab in the attached Exhibit which gives 11.82% as the yearly WACC.

c. **Pro-Forma Projection, FCF Valuation**

The pro-forma model is found in the complementary Excel spreadsheet in the ‘Pro-Forma’ tab. In it, there is previous five years of quarterly financial data given as inputs in blue. All black colored values are formulas or calculations. It projects out the next 20 quarters using the assumptions in red at the top. These assumptions were built from historical average rates, expected future trends, and analyst reports. These projections are then brought to the ‘FCF Valuation’ tab where the actual cash flows to the share-holders are built. A terminal value is estimated by assuming a terminal value growth rate of 2.75% per quarter. The formula for a perpetuity is then used to calculate a terminal value that theoretically includes all of the firm’s future cash flows beyond the projection period. This estimate is reasonable as it is significantly less than the current high-growth rate of 9.66% per quarter and in my opinion can be sustained given the high rate of investment into R&D that should keep Vertex with at least some product revenue growth going forward. However, it is difficult to pin down an exact value of the terminal growth rate without knowing something about the future state of the company which in biopharma is of course, highly uncertain. Because the terminal value represents such a large portion of a firm’s total value (assuming it will continue operating indefinitely) it is extremely sensitive to the growth rate. To help with this problem, a data table of sensitivities is displayed further down on the ‘FCF Valuation’ tab. This shows a range of discount rates and various terminal growth rates and which combinations lead to an implied share price greater than the market price (in green) and which are lower (in red).
All of these projected cash flows and the terminal value are then discounted and summed to arrive at an estimated value of Vertex’s current company.

d. **SYMDEKO® Valuation**

The recently approved SYMDEKO® has now been approved and is valued according to a DCF because the market risk is low and technical risk has already been surpassed because it was able to advance through the development process. It showed superior efficacy and safety profiles in comparison to other treatments in the clinic. Careful consideration is given to cannibalization effects for the projections of future revenues of KALYDECO® some of which will be taken by SYMDEKO®. Attention is also given to the patent timeline and competitors who have treatments in the development process, which if approved, would disrupt Vertex’s current monopoly and potentially create a large loss of revenues compared to the projections. The main factors are all built into the model and presented on the ‘SYMDEKO’ tab in the Exhibit. Launch costs are assumed to be similar to those of the previous products at around $200 million. The net price assumes that 30% of the sale price is used for manufacturing. The total theoretical population value comes from research commenting on how many previously untreated patients are expected to be treated with SYMDEKO®. Also, it includes the number that may switch from another product to SYMDEKO® for the reduced side-effects and better efficacy. The accessible population rate of 85% assumes that there is some level of underinsurance where patients are unable to gain access to the drug. This is defended by the slower than expected reimbursement coverage for ORKAMBI® in certain EU countries like France where Vertex has been selling the drug but have not recorded any revenues due to broken negotiations over the price that the French government will accept. The uptake trend figures follow that of the previously launched drugs as well. The
total NPV of this product according to the assumptions made here is $5.69 Billion. This is extremely significant for the company and signals that if adoption goes as planned this will be yet another blockbuster product for Vertex.

e. Triple Combination Therapy Valuation, Application of Monte Carlo Simulation

This analysis uses the MCS-GBM (standard Monte Carlo) model to evaluate the triple combination therapy based on assumptions regarding price, penetration (especially access in the EU), longevity, success at stage of development, and approval.

The assumption for the cumulative probability of positive Phase 3 clinical data and the rate of NDA approval (if submitted) is taken from Dimasi et al. and Hay et al. and then adjusted upwards by 10% to 69% in order to account for this specific treatment’s status as combination therapy in which two of the underlying drugs have already been approved. This gives stronger inclination for the regulatory authorities to approve this therapy.\footnote{DiMasi, J.A et al, 2010} Only between 10-25% of drugs ever achieve cash flows past the pre-registration phase (and hence are never positive NPV and should be rejected as projects).\footnote{Hay et al, 2014} However, this triple combination therapy has already surpassed the riskiest parts of the drug development process.

Anthony Walker from the consulting group, Alacrita, suggests a using risk-profiled NPV (rpNPV) to value pipeline products which as described in his paper is the most accurate to reality, especially when run with stringent constraints. That type of analysis generates a tri-modal distribution showing a large probability of loss, medium probability of breakeven and the very low

\footnote{DiMasi, J.A et al, 2010}
\footnote{Hay et al, 2014}
\footnote{Walker et al, 2015}
probability of winning big. This is the preferred method to view an early stage pipeline product, however, in this case, it is unnecessary to analyze the triple-combination therapy in this way due to it already beginning Phase 3 trials.

Instead, a simpler Monte Carlo simulation similar to the one his paper first presents was performed with 50,000 trials. This is far more practical for the development stage that the drug us in. The assumptions for this projection are presented on the ‘Triple-Combo’ tab in the Exhibit. These assumptions were built off of research, analyst reports and values achieved by the previously launched products. The input distribution ranges are also presented in the Exhibit with most following a PERT distribution which allows for entering a smallest, largest and likeliest value and is a smoothed form of a triangular distribution. The simulation returned a mean NPV of about $6.6 Billion.

f. Results

The entire outcome distribution and the associated statistics are presented in the following figures:
Frequency Distribution of rNPV at 90% Confidence Interval, Source: Own Creation

Summary Statistics of MC Simulation, Source: Own Creation
The chart labeled sensitivity, also known as a Tornado chart, shows which input variables explain which portion of the variation in the outcomes. As seen here, cumulative Market Share, Accessible Population and Net Price are the most important factors in the determination of NPV for the new product. This reinforces the recommendations derived from the business analysis frameworks presented in Analysis I; Minimization of effects from competition, Market Access and Pricing. The implementation of those strategies does much to reduce the variability around the inputs and thus gives a more certain and often larger return.
XII. Target Stock Price

The implied stock price in this model is calculated by summing the three discounted cash flows of the firm and subtracting the value of debt and adding cash. Then this is divided by the number of shares outstanding to get an implied share price. As previously stated, the enterprise value here involves the sum of the projected five years, the terminal value, and the two pipeline products covered in this analysis. The total results are summarized in the table on the following page. The implied share price in the base case scenario (all assumptions as given) is **$171.94**. This represents a 6.82% increase over the current market share price of $161.89 (as of day close April 12, 2018). The section labeled ‘Monte Carlo Mean’ uses the mean value from the MCS of the triple-combination product in the calculations. This gives a similar result of 6.34% greater.

The sections labeled ‘Monte Carlo Low’ and ‘Monte Carlo High’ repeat the same calculations instead using the low and high values of the 90% confidence interval around the mean of the simulation distribution. In the low scenario the implied price is 3.86% higher and 9.00% higher for the high scenario.
### Projected Price & Return

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<td>Plus: Cash &amp; Equivalents</td>
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<td>6.82%</td>
<td>6.34%</td>
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35
a. Investment Recommendation

Vertex has extremely strong market potential and is a hot stock to trade due to the strong status of these future projections. It is even more compelling because as the cash flows from revenue increase the financial standing of the firm increases and they can afford to either pay it out as dividends to shareholders or reinvest in new projects. Based on this analysis, I recommend VRTX as a buy. I would upgrade this recommendation to a strong buy if the Phase 3 data from the Triple-combination therapy starts coming back positively. It is possible that this product will show more efficacy than thought and increase its value.

XIII. Limitations

This model has limitations in several areas. Mostly, these are in the assumptions being made based on historical rates. There is no indication that historical trends will continue into the future, so the model must make a defendable assumption and either use that value or update it according to new research or trends. Secondly, the estimates for the discount rate and terminal growth rate are very sensitive and small adjustments to these dramatically change the price. As part of this, it was assumed that Vertex would keep almost the same capital structure where they are financing with around 40% debt, however, this value could change in the future as the company may seek to lever up to benefit from interest tax shields now that they are paying taxes due to positive net incomes for the first time. Thirdly, the projections for SYMDEKO® were assumed to be true for simplicity, but a deeper analysis would start to look at initial data of product sales and estimate the actual level of uptake in each year as well as looking at sensitivities around those input variables. Lastly, there is a slight limitation in the Monte Carlo Simulation in that it may not fully account for the true variability in inputs because they were made based on assumptions of the previous drug launch, ORKAMBI®. It also does not account for extremely adverse events that are outliers, but in a real scenario could drastically affect the projected NPV value.
XIV. Future Considerations

If Vertex is able to successfully bring SYMDEKO® and the Triple-Combination therapy to market as well as grow the existing ones they will have a large inflow of cash. This causes them to be a great candidate for making a strategic acquisition given the need to also de-risk away from only CF. The firm currently has a partnership with CRISPR Therapeutics which has a gene editing platform that would cure genetic diseases by splicing out pieces of mutated DNA and replacing it with the correct sequence so that the cells grow, and the individual is healthy. Vertex is looking to treat sickle cell disease and Beta-Thalassemia with this which are both blood diseases caused by mutated proteins. As such, they may look to make an acquisition of IP or expert research groups in this space. It is interesting to note that sickle cell disease is primarily found people of Sub-Saharan African descent in comparison with CF which is primarily Caucasian. This would serve not only to diversify the products, but also open up to new areas of financing or advocacy groups with a particular interest in investing funds to help people of African descent.

Maybe an even better target would be to go after another disease in an organ, like the kidney. On the Vertex partnerships page, they mention they are seeking potential collaborators to perform research on Polycystic Kidney Disease (PCD). From initial research, this target seems to have the same type of mutation leading to a defective protein as does CF. Both of these disease targets fit with the Vertex strategy. The beauty of their business model is in focusing on ‘rare’ disease that sell themselves thus reducing sales force expenditures, gain high-reimbursement prices because the individuals are so extremely sick, and require little capital expenditure to produce. All that is needed is R&D spending which is their specialty given the history as a research focused organization. The unique financing model of accepting funds from an advocacy group is risky in that you may be pressured to research only in one particular area even against the better judgement of the firm as a whole, but the terms are likely to very favorable with little interest on debt and only paying milestones in the pipeline advances. It is yet to be seen if Vertex will try this model again or if other firms may follow their example.

Vertex themselves could also be a candidate for acquisition by a large pharmaceutical. The acquiring company may want to purchase the significant CF assets that Vertex has and would likely pay a
premium for the company. This value would be based on the expected price of Vertex, plus any additional value of synergies that the acquiring company would receive. However, this may not be the best option because of Vertex’s unique culture there may be difficulties integrating with a parent company. Part of the Vertex identity is the ability to have autonomy and work on developing truly innovative medicine.

In all, the company should seek to continue to provide access for eligible individuals that can benefit from their life-altering products. The focus for companies to succeed in modern times is to adhere to a triple-bottom line philosophy where returns are measured not only in economic profits, but in social and environmental gains as well. A strong biotech such as Vertex has a tremendous opportunity to operate in such a manner because they are bettering the lives of chronically ill people and their families.
XV. Acknowledgements

This project has been an amazing learning experience and test of mental and physical fortitude. It was made much easier with the help of my primary reader, Dan Bergstresser and the insightful advice of my secondary reader, Bhoomija Ranjan. Also, thank you to Hagit Weihs for guiding the project to its completion.

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Hamza Abdurezak, Professor of Finance – Brandeis International Business School
XVI. References


